

## Claims

1. A method for identifying a compound that modulates a EFEMP1 bioactivity, comprising the steps of:
  - (a) contacting an appropriate amount of the compound with a cell or cellular extract, which expresses an EFEMP1 gene; and
  - (b) determining the resulting EFEMP1 bioactivity, wherein an increase or decrease in the EFEMP1 bioactivity in the presence of the compound as compared to the bioactivity in the absence of the compound indicates that the compound is a modulator of an EFEMP1 bioactivity.
2. A method of claim 1, wherein the EFEMP1 gene is a human EFEMP1 gene.
3. A method of claim 1, wherein the EFEMP1 gene is a wildtype gene.
4. A method of claim 1, wherein the EFEMP1 gene is a mutant gene.
5. A method of claim 1, wherein the modulator is an agonist of an EFEMP1 bioactivity.
6. A method of claim 1, wherein the modulator is an antagonist of an EFEMP1 bioactivity.
7. A method of claim 1, wherein in step (b), the EFEMP1 bioactivity is determined by determining the expression level of an EFEMP1 gene.
8. A method of claim 7, wherein the expression level is determined by detecting the amount of mRNA transcribed from an EFEMP1 gene.
9. A method of claim 7, wherein the expression level is determined by detecting the amount of EFEMP1 gene product produced.
10. A method of claim 9, wherein the expression level is determined using an

anti-EFEMP1 antibody in an immunodetection assay.

11. A method of claim 1, which additionally comprises the step of preparing a pharmaceutical composition from the compound.

12. A method of claim 1, wherein said cell is contained in an animal.

13. A method of claim 12, wherein the animal is transgenic.

14. A method of claim 13, wherein the transgenic animal contains a human EFEMP1 gene.

15. A compound identified by the method of claim 1.

16. A compound of claim 15, which is selected from the group consisting of: a small molecule, a polypeptide, a nucleic acid and a peptidomimetic.

17. A compound of claim 16, wherein the nucleic acid is selected from the group consisting of: an antisense molecule, a ribozyme and a triplex nucleic acid.

18. A compound of claim 16, wherein the polypeptide is a EFEMP1 polypeptide.

19. A method for identifying whether a test molecule is an EFEMP1 binding partner or measuring the strength of an interaction between an EFEMP1 polypeptide and said EFEMP1 binding partner comprising:

- (a) allowing (i) a first molecule comprising a EFEMP1 polypeptide operably linked to a heterologous DNA binding domain to interact with (ii) a second molecule comprising a test molecule operably linked to a polypeptide transcriptional activation domain and (iii) a hybrid reporter gene comprising a nucleic acid encoding a reporter operably linked to a DNA sequence comprising a binding site for said heterologous DNA binding domain; and
- (b) detecting or measuring the expression of the hybrid reporter gene as an indication of the existence or strength of an interaction between the first molecule and the second molecule wherein high levels of hybrid reporter expression indicate a strong interaction between EFEMP1

and said test molecule thereby identifying a test molecule which is an EFEMP1 binding partner.

20. A method of claim 19, wherein said second molecule is encoded by a nucleic acid and comprises a test polypeptide operably linked to a polypeptide transcriptional activation domain, and which further comprises the step of isolating the nucleic acid encoding said second molecule from a cell expressing the hybrid reporter gene.

21. A method for identifying a molecule which is a downstream or an upstream component of an EFEMP1 biochemical pathway or for measuring the strength of the interaction between a EFEMP1 biochemical pathway component and a EFEMP1 binding partner comprising:

(a) allowing (i) a first molecule comprising a EFEMP1 binding partner polypeptide operably linked to a heterologous DNA binding domain to interact with (ii) a second molecule comprising a test molecule operably linked to a polypeptide transcriptional activation domain and (iii) a hybrid reporter gene comprising a nucleic acid encoding a reporter operably linked to a DNA sequence comprising a binding site for said heterologous DNA binding domain; and  
(b) detecting or measuring the expression of the hybrid reporter gene as an indication of the existence or strength of an interaction between the first molecule and the second molecule wherein high levels of hybrid reporter expression indicate a strong interaction between a EFEMP1 binding partner and said test molecule thereby identifying a test molecule which is a downstream or an upstream component of the EFEMP1 biochemical pathway.

22. A method of claim 21, wherein said second molecule is encoded by a nucleic acid and comprises a test polypeptide operably linked to a polypeptide transcriptional activation domain, and which further comprises the step of isolating the nucleic acid encoding said second molecule from a cell expressing the hybrid reporter gene.

23. A method for identifying a compound, which interacts with a EFEMP1 polypeptide or EFEMP1 binding partner, comprising the steps of:

(a) contacting an appropriate amount of the compound with a EFEMP1 polypeptide and a EFEMP1 binding partner under conditions wherein, but for the test compound, the EFEMP1

polypeptide and EFEMP1 binding partner are able to interact; and

(b) detecting the extent to which a EFEMP1 polypeptide/EFEMP1 binding partner complex is formed in the presence of the compound, wherein an increase or decrease in the amount of complex formed in the presence of the compound relative to in the absence of the compound indicates that the compound interacts with a EFEMP1 polypeptide or EFEMP1 binding partner.

24. A method of claim 23, wherein the EFEMP1 polypeptide is a human EFEMP1 polypeptide.

25. A method of claim 23, wherein the EFEMP1 polypeptide is a wildtype polypeptide.

26. A method of claim 23, wherein the EFEMP1 polypeptide is a mutant polypeptide.

27. A method of claim 23, wherein the compound, which interacts with a EFEMP1 polypeptide or EFEMP1 binding partner is a EFEMP1 agonist.

28. A method of claim 23, wherein the compound, which interacts with a EFEMP1 polypeptide or EFEMP1 binding partner is a EFEMP1 antagonist.

29. A method of claim 23, which additionally comprises the step of preparing a pharmaceutical composition from the compound.

30. A compound identified by the method of claim 29.

31. A compound of claim 30, which is selected from the group consisting of: a small molecule, a polypeptide, a nucleic acid and a peptidomimetic.

32. An isolated EFEMP1 nucleic acid which is operably linked to a EFEMP1 transcriptional regulatory sequence.

33. A nucleic acid of claim 32, wherein the EFEMP1 transcriptional regulatory sequence is selected from the group consisting of: a EFEMP1 enhancer, a EFEMP1 promoter, and a EFEMP1 initiator element.

34. An isolated nucleic acid of claim 32, wherein the EFEMP1 nucleic acid is functionally fused to a heterologous gene.

35. An isolated nucleic acid of claim 34, wherein said heterologous gene encodes a protein selected from the group consisting of: a positive selectable marker, a negative selectable marker and a reporter gene.

36. An isolated nucleic acid of claim 35, wherein the coding sequence of EFEMP1 is disrupted by a positive selectable marker.

37. An isolated nucleic acid of claim 36, wherein said nucleic acid is further flanked by a negative selectable marker or markers.

38. An isolated nucleic acid of claim 37, wherein the reporter gene is selected from the group consisting of: beta-galactosidase and luciferase.

39. A cell line comprising an isolated nucleic acid of claim 39.

40. An animal comprising an isolated nucleic acid of claim 39.

41. An animal of claim 40, which is transgenic.

42. An animal of claim 41, which contains a human EFEMP1 gene.

43. An isolated nucleic acid comprising a EFEMP1 responsive regulatory sequence operably linked to a reporter gene.

44. An isolated nucleic acid of claim 43, wherein the reporter gene is selected from the group consisting of: beta-galactosidase and luciferase.

45. A cell line comprising an isolated nucleic acid of claim 43.
46. An animal comprising an isolated nucleic acid of claim 43.
47. An animal of claim 46, which contains a human EFEMP1 gene.
48. A cell in which the biological activity of one or more EFEMP1 proteins is altered by a chromosomally incorporated transgene.
49. A cell of claim 48, wherein said transgene disrupts at least a portion of a genomic EFEMP1 gene.
50. A cell of claim 48, wherein said transgene deletes all or a portion of the genomic EFEMP1 gene by replacement recombination.
51. A cell of claim 48, wherein said transgene comprises: (i) at least a portion of the genomic EFEMP1 gene, and (ii) a marker sequence which provides a detectable signal for identifying the presence of the transgene in a cell.
52. A transgenic animal comprised of a cell of claim 48.